

**TESTIMONY OF CRAIG C. MELLO, PhD**

***2006 NOBEL LAUREATE IN PHYSIOLOGY or MEDICINE  
for the DISCOVERY of RNA INTERFERENCE***

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**BEFORE THE SENATE COMMITTEE ON COMMERCE, SCIENCE AND TRANSPORTATION**

**ON  
RNA INTERFERENCE**

**MAY 2, 2007**

Good afternoon. Thank you, Congressman McGovern, for that kind introduction.

Mr. Chairman and members of the committee, it is a privilege to have the opportunity to testify before you this afternoon.

In a small lab at the University of Massachusetts Medical School and a small lab at the Carnegie Institution of Washington, with support from the NIH and other private sources, Andy Fire and I made a series of observations that have sparked a revolution in our understanding of how the genetic information that makes us human is stored and expressed inside our cells. Today, as we speak, thousands of scientists in labs all over the world are building on these discoveries to understand and to develop treatments for human disease, to shed further light on the basic functioning of cells, and to study and modify plants, animals and microbes important in agriculture, biofuels and other applications essential to meeting the many needs of our civilization.

Mr. Chairman, members of the committee, we as a nation, indeed we humans as a species, are dangerously out of equilibrium with our environment. Pressures from over-population and lack of quality medical care in third-world countries (and even here in the US) are leading to millions of unnecessary deaths each year, deaths from diseases we know how to treat, and these medically underserved populations are incubating new, potentially devastating pathogens. Alternative fuels and better crops must be developed to support populations that have already reached sizes that challenge the very productive capacity of the planet. In short, we need a call to arms, a call to fund science broadly in this country so that our nation can face these challenges and can continue to lead the world toward a brighter future.

The discovery of gene silencing by double-stranded RNA—"RNA interference," or "RNAi," for which Andy and I were awarded the 2006 Nobel Prize in Physiology or Medicine—was not something that anyone was looking for. We knew, based on some early and unexpected laboratory observations, that there was something puzzling going on, and we grew more excited over time by what we were seeing as we tried to understand. RNA interference went from being a puzzle, to being understood well enough for us to publish a paper in the prestigious journal *Nature* in 1998, to being applied as a tool for treating human disease, to being recognized with the Nobel Prize, in just eight years. The research and the discovery were all the more exciting to us because it was all so unexpected.

This could happen only because we are in an era unprecedented for the potential for scientific discovery. The investments in science made in the late 1990s and the first years of this century opened vast opportunities for science and scientists: universities built research labs and trained and hired new young scientists—like myself and Andy—who in turn made new contributions that other scientists learned from and expanded upon. The investments in facilities and training and the tools of research were the investments that led to the sequencing of the human genome—the mandatory first step in realizing the dream of interfering with disease at the genetic level. RNAi has tremendous promise for building on the work of the Human Genome Project, but only if further research is funded and allowed to continue. Importantly, information, the universal currency of science, now flows effortlessly and almost instantly around the globe. Consequently, the pace of discovery is picking up worldwide, increasing the opportunities for discovery but also increasing the competition for US laboratories. If we do not increase the US investment to keep pace with these opportunities, then we will see future multibillion dollar technologies like RNAi discovered and developed abroad. If we don't act now to increase science funding, other countries will capitalize on the investments we, the American people, have made in funding science over the past decades.

At the University of Massachusetts, we have established an RNAi Therapeutics Center to further capitalize on this momentum and our own particular expertise in the field of RNAi-based gene silencing. The vision for this Center emphasizes facilitating and promoting clinical and translational research and ultimately developing the next generation of powerful drugs to treat a broad range of diseases including cancers, Alzheimer's, diabetes heart disease, and many other areas in which my renowned UMass colleagues have already dedicated years of work.

At UMass, there is a strong belief that science, and research, do truly matter, for a much larger reason than prizes or prestige: science matters because no one knows from where, or how, or based on what unpredictable series of events, the next breakthrough might come, and there's never been a moment in human history with more opportunity or greater need

for advances in the life sciences than right now. This isn't science for the sake of science, but science for the sake of medical advances and lives to be saved.

This is just the beginning! The confluence of the energetic students and innovative young scientists trained in the last two decades, with the investment in facilities and resources, combined with the discoveries of the past few years, all flow together to create a perfect moment of opportunity. But just at the time when we should be investing in science at an unprecedented level, we are not. Just at the moment when we should be capitalizing on the investments of the past decade, funding for basic research is in decline. If Andy and I had been faced with today's funding climate 10 years ago when we applied for support for the work that led to the discovery of RNAi, I don't think we would have received that support. What other discoveries—what work like RNAi, what research that will advance it in ways we can't even imagine—will be missed, because we stepped back from the opportunity?

Thank you. I will be happy to take your questions.